

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization International Bureau



(43) International Publication Date
15 September 2005 (15.09.2005)

PCT

(10) International Publication Number
WO 2005/084158 A2

(51) International Patent Classification: Not classified

(21) International Application Number: PCT/US2004/020837

(22) International Filing Date: 18 June 2004 (18.06.2004)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data: 60/480,065 20 June 2003 (20.06.2003) US

(63) Related by continuation (CON) or continuation-in-part (CIP) to earlier application:

US 60/480,065 (CON)

Filed on 20 June 2003 (20.06.2003)

(71) Applicant (for all designated States except US): THE REGENTS OF THE UNIVERSITY OF CALIFORNIA [US/US]; 1111 Franklin Street, Oakland, CA 94607-5200 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): DOWDY, Stephen,

F. [US/US]; 5761 Waverly Avenue, La Jolla, CA 92037 (US). WADIA, Jehangir, S. [CA/US]; 3205-12 Via Alicante, La Jolla, CA 92137 (US).

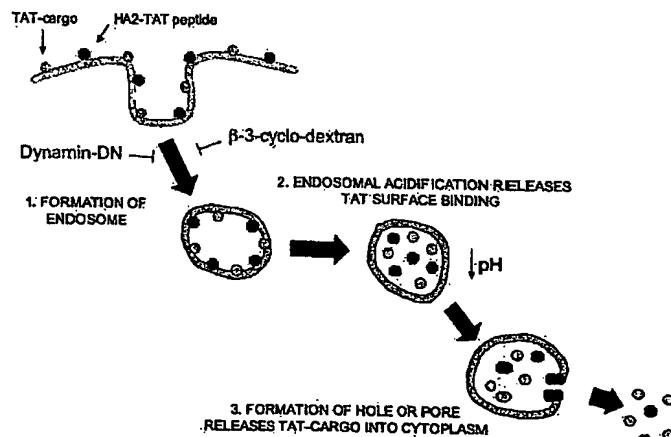
(74) Agent: BAKER, Joseph, R., Jr.; Fish & Richardson P.C., 12390 El Camino Real, San Diego, CA 92130 (US).

(81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US (patent), UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI,

[Continued on next page]

(54) Title: POLYPEPTIDE TRANSDUCTION AND FUSOGENIC PEPTIDES



WO 2005/084158 A2

(57) Abstract: Due to the barrier imposed by the cell membrane, delivery of macromolecules in excess of 500 Daltons directly into cells remains problematic. However, proteins, which have been evolutionarily selected to perform specific functions, are therefore an attractive therapeutic agent to treat a variety of human diseases. In practice, the direct intracellular delivery of these proteins has, until recently, been difficult to achieve due primarily to the bioavailability barrier of the plasma membrane, which effectively prevents the uptake of the majority of peptides and proteins by limiting their passive entry. However, recent work using small cationic peptides, termed protein transduction domains (PTDs), derived from polynucleotide binding proteins, such as HIV TAT protein or the Drosophila transcription factor Antp. or synthetic poly-Arginine, have now been shown to deliver a myriad of molecules, including synthetic small molecules, peptides and proteins, into animal models *in vivo*.



SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

Published:

- *without international search report and to be republished upon receipt of that report*